

(12) INTERNATIONAL APPLICATION PUBLISHED UNDER THE PATENT COOPERATION TREATY (PCT)

(19) World Intellectual Property  
Organization  
International Bureau



(43) International Publication Date  
12 August 2004 (12.08.2004)

PCT

(10) International Publication Number  
**WO 2004/067728 A3**

(51) International Patent Classification<sup>7</sup>: **C12Q 1/68**

(21) International Application Number:  
PCT/US2004/000423

(22) International Filing Date: 9 January 2004 (09.01.2004)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:  
60/441,028 17 January 2003 (17.01.2003) US

(71) Applicant (for all designated States except US): **PTC THERAPEUTICS** [US/US]; 100 Corporate Court, South Plainfield, NJ 07080 (US).

(72) Inventors: **PELLEGRINI, Matthew, C.**; Bedminster, NJ (US). **TROTTA, Christopher, R.**; Somerset, NJ (US). **HUQ, Shah, I.**; Edison, NJ (US).

(74) Agents: **SCOLA, Daniel, A., Jr.** et al.; Hoffmann & Baron, LLP, 6900 Jericho Turnpike, Syosset, NY 11791 (US).

(81) Designated States (unless otherwise indicated, for every kind of national protection available): AE, AG, AL, AM,

AT, AU, AZ, BA, BB, BG, BR, BW, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NA, NI, NO, NZ, OM, PG, PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, SY, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, YU, ZA, ZM, ZW.

(84) Designated States (unless otherwise indicated, for every kind of regional protection available): ARIPO (BW, GH, GM, KE, LS, MW, MZ, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PT, RO, SE, SI, SK, TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).

**Published:**

- with international search report
- before the expiration of the time limit for amending the claims and to be republished in the event of receipt of amendments

(88) Date of publication of the international search report:  
14 April 2005

For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.

(54) Title: METHODS AND SYSTEMS FOR THE IDENTIFICATION OF RNA REGULATORY SEQUENCES AND COMPOUNDS THAT MODULATE THEIR FUNCTION

(57) Abstract: The present invention is directed to a non-cell based method for identifying RNA regulatory sequences involved in translational control. The method includes: combining a translational extract; an RNA test sequence; and a reporter mRNA under suitable conditions for translation of the reporter mRNA. The method also includes measuring the effect of the test sequence on the translation of the reporter mRNA, wherein a test sequence that modifies the translation of the reporter mRNA includes an RNA regulatory element. The invention also provides methods and systems for identifying test compounds that modulate the regulatory activity of an RNA regulatory sequence. In the method, an RNA regulatory sequence, which regulates translation of a reporter mRNA, is combined with a translation extract; the reporter mRNA; and at least one test compound. The method further includes measuring the effect of the test compound on the ability of the RNA regulatory sequence to regulate the translation of the reporter mRNA.



WO 2004/067728 A3

# INTERNATIONAL SEARCH REPORT

International application No.

PCT/US04/004231

## A. CLASSIFICATION OF SUBJECT MATTER

IPC(7) : C12Q 1/68  
US CL : 435/6

According to International Patent Classification (IPC) or to both national classification and IPC

## B. FIELDS SEARCHED

Minimum documentation searched (classification system followed by classification symbols)  
U.S. : 435/6

Documentation searched other than minimum documentation to the extent that such documents are included in the fields searched

Electronic data base consulted during the international search (name of data base and, where practicable, search terms used)  
Please See Continuation Sheet

## C. DOCUMENTS CONSIDERED TO BE RELEVANT

Category *	Citation of document, with indication, where appropriate, of the relevant passages	Relevant to claim No.
X	US 2002/0086299 A1 (OBOKATA ET AL) 04 July 2002 (04.07.2002), see entire document, especially page 3, column 2 to page 4, column 1, and Examples 1-3.	1-17, 31, 33
Y		18-30, 32, 34
Y	US 5,814,471 (WOOD) 29 September 1998 (29.09.1998), column 9, lines 32-47.	18-30, 32, 34

☐ Further documents are listed in the continuation of Box C.

☐ See patent family annex.

Special categories of cited documents:	
"A" document defining the general state of the art which is not considered to be of particular relevance	"T" later document published after the international filing date or priority date and not in conflict with the application but cited to understand the principle or theory underlying the invention
"E" earlier application or patent published on or after the international filing date	"X" document of particular relevance; the claimed invention cannot be considered novel or cannot be considered to involve an inventive step when the document is taken alone
"L" document which may throw doubts on priority claim(s) or which is cited to establish the publication date of another citation or other special reason (as specified)	"Y" document of particular relevance; the claimed invention cannot be considered to involve an inventive step when the document is combined with one or more other such documents, such combination being obvious to a person skilled in the art
"O" document referring to an oral disclosure, use, exhibition or other means	"&" document member of the same patent family
"P" document published prior to the international filing date but later than the priority date claimed	

Date of the actual completion of the international search

06 February 2005 (06.02.2005)

Date of mailing of the international search report

07 MAR 2005

Name and mailing address of the ISA/US

Mail Stop PCT, Attn: ISA/US  
Commissioner for Patents  
P.O. Box 1450  
Alexandria, Virginia 22313-1450

Facsimile No. (703) 305-3230

Authorized officer

erry A. McKelvey

Telephone No. 703-308-0196

# INTERNATIONAL SEARCH REPORT

International application No.

PCT/US04/00428

## Box No. II Observations where certain claims were found unsearchable (Continuation of item 2 of first sheet)

This international search report has not been established in respect of certain claims under Article 17(2)(a) for the following reasons:

1. ☐ Claims Nos.:  
because they relate to subject matter not required to be searched by this Authority, namely:
2. ☒ Claims Nos.: 35-39  
because they relate to parts of the international application that do not comply with the prescribed requirements to such an extent that no meaningful international search can be carried out, specifically:  
Please See Continuation Sheet
3. ☐ Claims Nos.:  
because they are dependent claims and are not drafted in accordance with the second and third sentences of Rule 6.4(a).

## Box No. III Observations where unity of invention is lacking (Continuation of item 3 of first sheet)

This International Searching Authority found multiple inventions in this international application, as follows:

1. ☐ As all required additional search fees were timely paid by the applicant, this international search report covers all searchable claims.
2. ☐ As all searchable claims could be searched without effort justifying an additional fee, this Authority did not invite payment of any additional fee.
3. ☐ As only some of the required additional search fees were timely paid by the applicant, this international search report covers only those claims for which fees were paid, specifically claims Nos.:
4. ☐ No required additional search fees were timely paid by the applicant. Consequently, this international search report is restricted to the invention first mentioned in the claims; it is covered by claims Nos.:

Remark on Protest ☐ The additional search fees were accompanied by the applicant's protest.  
☐ No protest accompanied the payment of additional search fees.

# INTERNATIONAL SEARCH REPORT

International application No.

PCT/US04/00423

## Continuation of Box II Reason 2:

the claims are drawn to compounds and use of the compounds that were identified using the claimed assay. Because the structure of compounds identified by the assay are not adequately supported by the description because the structure of the compounds are not set forth, the compounds themselves and the corresponding methods of use are accordingly not adequately supported. Without a description of the structure of the claimed compounds, no meaningful search related to the compounds can be performed.

## Continuation of B. FIELDS SEARCHED Item 3:

EAST (USPAT, PGPUB, EPO, JPO, Derwent databases), Dialog OneSearch (biotech databases)

search terms: rna, regulat?, translat?, in vitro, extract, assay?, test?, compound?, screen?, utr, reticulocyte lysate